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# Biotechnology and oncology: The promise and the challenges

**By Dennis N. Longstreet**

*Mr. Longstreet, President of Ortho Biotech, Raritan, NJ, presented his views of the future for oncology and biotechnology. This article contains a complete transcript of Mr. Longstreet's remarks.*

We've spent this afternoon discussing how changes here in Washington will impact patient care—perhaps even in the next 100 days. You know that the President is serious, particularly if he has put his wife, someone whom he knows and trusts the most, in charge of health care reform.

It's more clear than ever that the medical community, government, and industry must join together and influence these changes. By working together, we can ensure that we bring the best treatments to patients. And the need for this partnership is even stronger as we look toward the future. Research laboratories across the country are developing exciting new therapies... many from the emerging science of biotechnology. But these scientific advancements have medical challenges which may be as complex as the science itself. Issues such as the cost of new product development, regulatory requirements, and reimbursement are creating challenges for the biopharmaceutical industry, for you, and your patients.

We can only meet these challenges by sharing our knowledge and expertise, and understanding each other's issues. Dialogues such as today's will help bring exciting scientific innovations to patients. Tonight, I'd like to look at the present and future of biotechnology, and open a dialogue which helps mesh together these exciting discoveries with better patient care.

First, let's look at how the science of biotechnology is impacting patient care, particularly in oncology. As you know, there has been significant progress in understanding the basis of human cancer. For example, we now know that oncogenes, tumor suppressor genes, and key genetic events are important targets which may revolutionize cancer therapy. Promising biological approaches to cancer therapy now include recombinant drugs, monoclonal antibodies, synthetic peptides and gene therapy, to name just a few. As an article in the March 1992 *Lancet* concludes, "The transition from theoretical possibility to practical reality is just beginning, and we could be at the dawn of a new age of cancer treatment."

Dennis Longstreet

***"Perhaps together, we can help promote policy change that deals with these reimbursement difficulties, and make therapies available to greater numbers of cancer patients"***

While practical applications of biotechnology to oncology therapy from your point of view have been painstaking and not without disappointment, they are under way. A new era of cancer therapy is not only possible, but probable. Eighteen licensed biotechnology medicines are now available: six for the treatment of cancer and cancer-related conditions. That is real progress since the industry introduced its first product just 10 years ago. Biotechnology's impact will be particularly important in oncology. Of the more than 130 biotechnology drugs currently in human clinical trials, greater than 50 percent are for cancer or cancer-related conditions. And this is only the shorter-term picture.

And as we look even further to the future, one of the most promising approaches for oncology is gene therapy. Of the 18 clinical protocols for human gene therapy trials approved by the FDA and the NIH Recombinant-DNA Advisory Committee, more than half target cancer. Gene therapy is not only technically feasible, but it also makes sense from a business and health care delivery standpoint. First, the need is great for this technology: many patient groups can be helped. It offers long-term treatment for several otherwise fatal diseases—diseases now managed at best only by costly conventional therapies. Many therapeutic approaches are possible from gene therapy. These include immune response activation, tumor cell destruction, and enhancement of conventional chemotherapy. Last, but certainly not least, we believe reimbursement is likely for several reasons, in part because we can work together to facilitate reimbursement. Let me explain this further.

There is a real likelihood that gene therapy will be superior to conventional therapies. There is even a possibility that it can be a one-time cure. We should have clear data on safety and efficacy, data which you can use to facilitate reimbursement for your patients. Working together, we must determine appropriate use of gene therapy, and demonstrate that these products have a favorable economic and clinical outcome. As industry, we must develop acceptable pricing for these products. While gene therapy and other approaches hold tremendous promise, we all recognize that cancer therapy development has been slow over the past decade.

So how realistic are predictions that these new therapies will be successful?

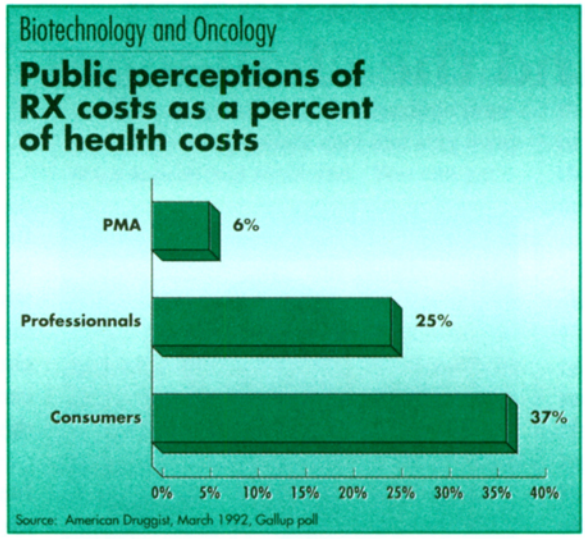
Continuing my gene therapy example, the first gene therapy recipients were treated more than two years ago. Two little girls with ADA deficiency successfully had that enzyme-expressing gene restored. Frequent reports in the medical literature document continued progress. As a matter of fact, this ADA experiment was done by Steven Rosenberg and his colleagues at NIH. Gene therapy is not just the future; it's today.

Also keep in mind that much of the human genome may be mapped out and sequenced in the next decade. We will be able to compare the genetic makeup of abnormal cells with their normal counterparts. The implications for cancer therapy are enormous.

By targeting the disease process, opportunities for discoveries are endless. An example is the new Ortho Biotech drug, Leustatin—OR 2-CdA. While not a biologic, it is an example of how rational drug design, which targets the molecular processes underlying the disease, is key to advances against cancer. Leustatin is awaiting approval from the FDA for treatment of hairy-cell leukemia, and we're excited by the new treatment options created by this compound.

The progress and the future of biotechnology are encouraging. But making these products available for patient care is not without its challenges—challenges which the biopharmaceutical industry can meet only by working together with the medical community and government. These challenges go far beyond the science itself. Many are related to the high cost of product development. Others are regulatory hurdles for obtaining FDA approvals, and difficulties in getting products reimbursed by third-party payers.

Let's look more in depth at these challenges—and keep in mind that I'm here tonight to give you an industry perspective. Because of the accelerating pace of scientific developments, the first challenge is choosing which drug to develop. This is a big decision since the cost of bringing a new drug to market is over $350 million dollars, on the average, according to a Tufts University study. Also, consider the industry's odds for success. A recent Duke University study showed that only 3 of every 10 pharmaceuticals launched from 1970 to 1979 recovered their research and development costs. Obviously, pharmaceutical companies make a tremendous upfront investment in drug development, and with biotechnology products, this investment decision can be even larger since it's made at the very beginning of a drug's development. These are first of a kind therapies. It's not as simple as deciding to modify an existing product, as is often the case with traditional pharmaceuticals.




The investment decision is also influenced by patent status. These decisions are made more difficult by the lag time in the U.S. Patent Office. Several thousand biotech patent applications await review, with an average review time of over two years. Without patent protection, you can invest in product development only to find yourself later involved in patent disputes.

Further clouding the picture, biotechnology patents are complex and not clearly defined. Until patent issues have been better resolved by our legal system, these uncertainties add risk to development decisions. Another factor in the development decision is the likelihood of reimbursement. As you all know, reimbursement is critical for the livelihood of your practice. And it's the same for the bio-pharmaceutical industry. With the significant R & D investment, we must feel confident that third-party payers will reimburse our products. Otherwise, we will never recover our development costs, much less make a profit.

Once we decide to move forward with a product, clinical development is the next challenge. Again, there's risk. For every new drug marketed, between six and seven fail during clinical development. Other considerations are likely to impact future clinical trials. Given the changing health care environment, insurance companies and managed health care providers are insisting on drug cost-effectiveness and outcomes data. It's also an increasing factor in development of HCFA reimbursement standards. This means— and rightfully so—that new therapies must demonstrate a favorable economic as well as clinical outcome. These data are critical for hospitals and managed care organizations to make formulary decisions.

Here's an example of how the biopharmaceutical industry and the medical community can work together. At Ortho Biotech, we initiated an outcomes management study with Procrit, our brand of recombinant erythropoietin. The study involves physicians at academic and community-based hospitals, as well as at managed care organizations. It evaluates the overall impact of Procrit therapy on a patient's quality of life, and measures other outcomes such as reductions in transfusions and hospitalization days.

We're also working with the medical community on another cost-effectiveness study for Procrit. This one seeks to determine the role of Procrit in enhancing the quality of life in Rye (RAI) Stage 3 and 4 Lymphocytic Leukemia patients. It's an EPO study with a definitive quality of life assessment—and again should gather important data for third-party payers and hospital formularies.

Together, we can go beyond economic analyses and begin developing appropriate usage guidelines for products. We can develop clinical trials which better identify patients who will most likely benefit from treatment. These appropriate usage guidelines ensure maximum patient benefit from therapy, and reduce costs by avoiding treatment for inappropriate patients.

More than ever, oncology clinical trials should be growing. More products are coming from biotechnology, and economic analysis studies are becoming increasingly important. But like you, we're concerned that reimbursement issues may threaten these trials. In some cases, insurers are denying payment for patient costs associated with clinical trials—jeopardizing important research which has long-term clinical, and even economic benefits. Again, we can join together on this issue and positively impact policy.

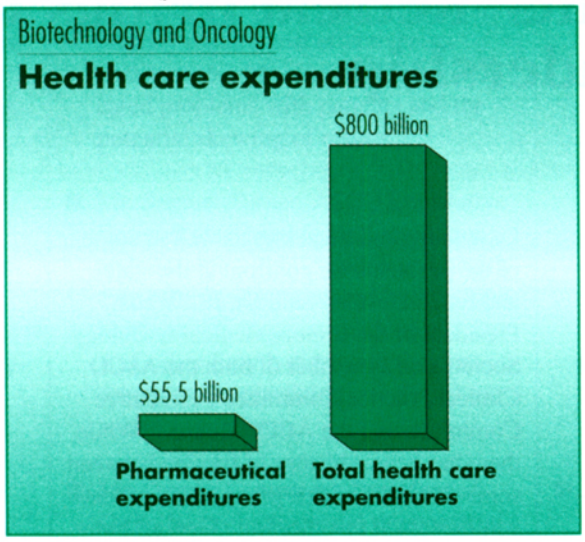
For the most part, the medical community and industry have a mutual understanding of clinical research. But you're probably less familiar with the regulatory challenges faced by the biotechnology

industry. This process is rigorous for traditional pharmaceuticals, but it is even more difficult for biotechnology products. With biologics, manufacturing establishments must be licensed, as well as the product. Our plants must meet demanding requirements and inspections before a drug can even begin production. Current regulations require that only one product can be produced in a biotech manufacturing facility. What's more, our commercial product must be manufactured at the same plant that supplies drug for clinical testing. This means that a manufacturer has a significant cost outlay long before it even knows if a drug will ever come to market—not only in establishing the plant, but in running it at far less than capacity while awaiting FDA licensing.

In addition, for most of the time before commercial distribution, the production process is frozen. After FDA approval, biologic manufacturers must have FDA pre-clearance for most manufacturing changes. That is not the case with traditional drugs, and this requirement for biologics virtually prevents improvements for quality and yield.

Then there's the lengthy review process of a new drug application or product licensing application. Current data from the Industrial Biotechnology Association show that the average time from drug discovery to approval is 7 to 10 years. Once clinical work is completed and submitted to the FDA, it takes about 22 months for the FDA to review the application. Furthermore, obtaining additional indications for products, even if they are already in clinical use, can take up to an estimated four years. Unfortunately, the time and dollar investment required for additional indications does discourage broader product applications. While the FDA has taken initiatives to speed-up the process, we await implementation. And we all know that this delay creates tremendous problems in off-label reimbursement—problems for physicians, patients, and manufacturers.

Here is an example of how industry and government can work together. Industry needs clear and consistent guidance as well as more timely response from all levels of the FDA. This partnership is critical to control development costs, make better products available for patient care, and ease reimbursement issues for industry and the medical community. Drug development and regulatory challenges seem simple as compared to reimbursement for these products. In an attempt to work with the medical community on these issues, many manufacturers have developed programs to facilitate reimbursement.




It's difficult to discuss reimbursement without getting into off-label issues. We're painfully aware of your difficulties with reimbursement. We have tried to develop programs which address your concerns. We welcome your feedback on how to improve our programs. This is another example of how working together can address critical issues for the oncology community as well as manufacturers. Perhaps together, we can help promote policy change that deals with these reimbursement difficulties, and make therapies available to greater numbers of cancer patients.

The ACCC efforts with state legislatures on off-label reimbursement are a perfect example of how we can together influence change. The ACCC has successfully combined the efforts of oncologists, oncology nurses, health care policy makers, HIV organizations, and even industry to pass legislation in several states—legislation which mandates reimbursement for off-label treatments. This is a promising sign of what we can accomplish by working together.

I recognize that you are probably concerned about the high costs of new innovations, particularly those from biotechnology. We've discussed the high development costs of these products. But I want to take our discussion a step further. Let's look at how these innovative products can actually decrease costs, and be part of the solution to rising health care expenditures. Projections over the next 25 years indicate that pharmaceuticals will save many more lives and billions of dollars, according to a recent study by the Batelle Medical Technology and Policy Research Center. Cancer was one of five groups of diseases analyzed in this study. For leukemia, lung, and colorectal cancer alone, the study projected that innovations in pharmaceuticals could avoid 155,000 cases and prevent 662,000 deaths—for a savings of $15.7 billion. Many of these cost-savings projections are based on assumptions that new biotechnology treatments will cure previously hopeless diseases, speed recovery, better manage chronic disease, and avoid side effects of treatment—assumptions which I believe are on target. These innovations will also promote cost savings by moving the site of care away from expensive acute settings.

Despite the facts, the public perceives that pharmaceuticals are responsible for a greater percentage of health expenditures than is actually correct. According to the Pharmaceutical Manufacturers' Association, drugs account for 6 percent of total health care costs. But in a recent Gallup survey, professionals believed that pharmaceuticals are responsible for 25 percent of expenditures, and consumers felt that drugs were 37 percent of total health care costs. A government analysis further shows that biotech and traditional pharmaceuticals are only a small percentage of total health care expenditures. A 1992 analysis shows that drugs accounted for only $55 billion of the nation's $800 billion health care bill. Why do these misperceptions exist? What does it mean? To me, this indicates that we must all work closer together to truly understand the issues and form an alliance for progress.

We must mount health care reform which identifies the real problems and avoids creating more difficulties for the future. We can't work in a vacuum. We need to learn about the issues which each of us face—government, the medical community, and industry. And we must keep the dialogue going. With a better understanding, we can avoid pitfalls, minimize mistakes, and forge a real alliance for progress.

Together, we can make positive changes—changes which recognize the future of sciences like biotechnology—and make scientific discoveries become treatment innovations. Together, we can reduce our total health care bill, yet provide improved patient care.